

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
12 May 2005 (12.05.2005)

PCT

(10) International Publication Number
WO 2005/042705 A2

- (51) International Patent Classification⁷: C12N (74) Agent: DELUCA, Mark; Cozen O'Connor, 1900 Market Street, Philadelphia, PA 19103 (US).
- (21) International Application Number: PCT/US2004/035124 (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (22) International Filing Date: 22 October 2004 (22.10.2004)
- (25) Filing Language: English
- (26) Publication Language: English
- (30) Priority Data:
60/513,489 22 October 2003 (22.10.2003) US (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- (71) Applicant (for all designated States except US): THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA [US/US]; 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): MOURELATOS, Zissimos [GR/US]; 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US). SHARMA, Anup [US/US]; 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US). KIRIAKIDOU, Marianthi [GR/US]; 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US).
- Published:
— without international search report and to be republished upon receipt of that report
- For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: SHORT INTERFERING RNA AND MICRO-RNA COMPOUNDS AND METHODS OF DESIGNING, MAKING AND USING THE SAME

(57) Abstract: Methods of identifying, designing and synthesizing uniquely targeting siRNA nucleotide sequences for a target mRNA sequence of a target species are disclosed. Methods of identifying, designing and synthesizing miRNA nucleotide sequences that does not function as a siRNA nucleotide sequence for mRNA of a target species are disclosed. Method of inhibiting expression of a target mRNA molecule are disclosed sequence. siRNA molecules including uniquely targeting siRNA molecules and RISCs comprising the same are disclosed. miRNA molecules that do not function as siRNA nucleotide sequences for mRNA of a target species are disclosed.

WO 2005/042705 A2